



[Billing Code 4140-01-P]

DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Prospective Grant of Exclusive Patent License: Development of adeno-associated virus-based vectors for the treatment of Menkes disease and related copper transport disorders

AGENCY: National Institutes of Health, HHS.

ACTION: Notice.

SUMMARY: The *Eunice Kennedy Shriver* National Institute of Child Health and Human Development, an institute of the National Institutes of Health, Department of Health and Human Services, is contemplating the grant of an Exclusive Commercialization Patent License to practice the inventions embodied in the U.S. Patents and Patent Applications listed in the Supplementary Information section of this notice to Cyprium Therapeutics, Inc. (“Cyprium”) located in New York, NY, USA.

DATES: Only written comments and/or applications for a license which are received by the National Cancer Institute’s Technology Transfer Center on or before [INSERT DATE 15 DAYS FROM DATE OF PUBLICATION OF NOTICE IN THE FEDERAL REGISTER] will be considered.

ADDRESSES: Requests for copies of the patent application, inquiries, and comments relating to the contemplated Exclusive Commercialization Patent License should be directed to: Surekha Vathyam, Ph.D., Senior Licensing and Patenting Manager, NCI Technology Transfer Center, 9609 Medical Center Drive, RM 1E530 MSC 9702, Bethesda, MD 20892-9702 (for business mail), Rockville, MD 20850-9702 Telephone: (240)-276-5530; Facsimile: (240)-276-5504 E-mail: vathyams@mail.nih.gov.

SUPPLEMENTARY INFORMATION:

Intellectual Property

United States Provisional Patent Application No. 62/244,594, filed October 21, 2015 and entitled “Codon-optimized Reduced-size ATP7A cDNA and Uses for Treatment of Copper Transport Disorders” [HHS Reference No. E-062-2015/0-US-01].

The patent rights in these inventions have been assigned and/or exclusively licensed to the government of the United States of America.

The prospective exclusive license territory may be worldwide and the field of use may be limited to the use of Licensed Patent Rights for the following: “Development and commercialization of adeno-associated virus-based vectors for the treatment of Menkes Disease and related copper transport disorders.”

This technology discloses a codon-optimized reduced-size Adenosine Triphosphate 7A (ATP7-alpha or ATP7A) cDNA, vectors, and recombinant adeno-associated viruses (AAVs) and uses thereof for treatment of copper transport disorders. Such uses, include the administration of copper in addition to ATP7A in order to maximize the advantage of the gene therapy.

Human P-type ATPase copper-transporting ATPase 1 (ATP7A) transports copper from enterocytes (where it is taken up from dietary copper) into the blood. ATP7A also mediates passage of copper across the blood-cerebrospinal fluid barrier and the blood-brain barrier. In Menkes disease and occipital horn syndrome (OHS), ATP7A activity is reduced or absent and copper export from the enterocytes is impaired. As a result, copper accumulates in intestinal cells and less copper is delivered to the blood, resulting in restricted copper supply to other tissues, particularly the brain. If successfully developed,

this invention would be a first of its kind therapy for treating copper transport disorders, such as Menkes disease, OHS, or ATP7A-related distal motor neuropathy, by administering the disclosed nucleic acid, vector, or recombinant virus to a subject with a copper transport disorder.

This notice is made in accordance with 35 U.S.C. 209 and 37 CFR Part 404. The prospective Exclusive Commercialization Patent License will be royalty bearing and may be granted unless within fifteen (15) days from the date of this published notice, the National Cancer Institute receives written evidence and argument that establishes that the grant of the license would not be consistent with the requirements of 35 U.S.C. 209 and 37 CFR Part 404.

Complete applications for a license in the prospective field of use that are filed in response to this notice will be treated as objections to the grant of the contemplated Exclusive Commercialization Patent License Agreement. Comments and objections submitted to this notice will not be made available for public inspection and, to the extent permitted by law, will not be released under the *Freedom of Information Act*, 5 U.S.C. 552.

Dated: September 21, 2016.

Richard U. Rodriguez,
Associate Director
Technology Transfer Center
National Cancer Institute

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